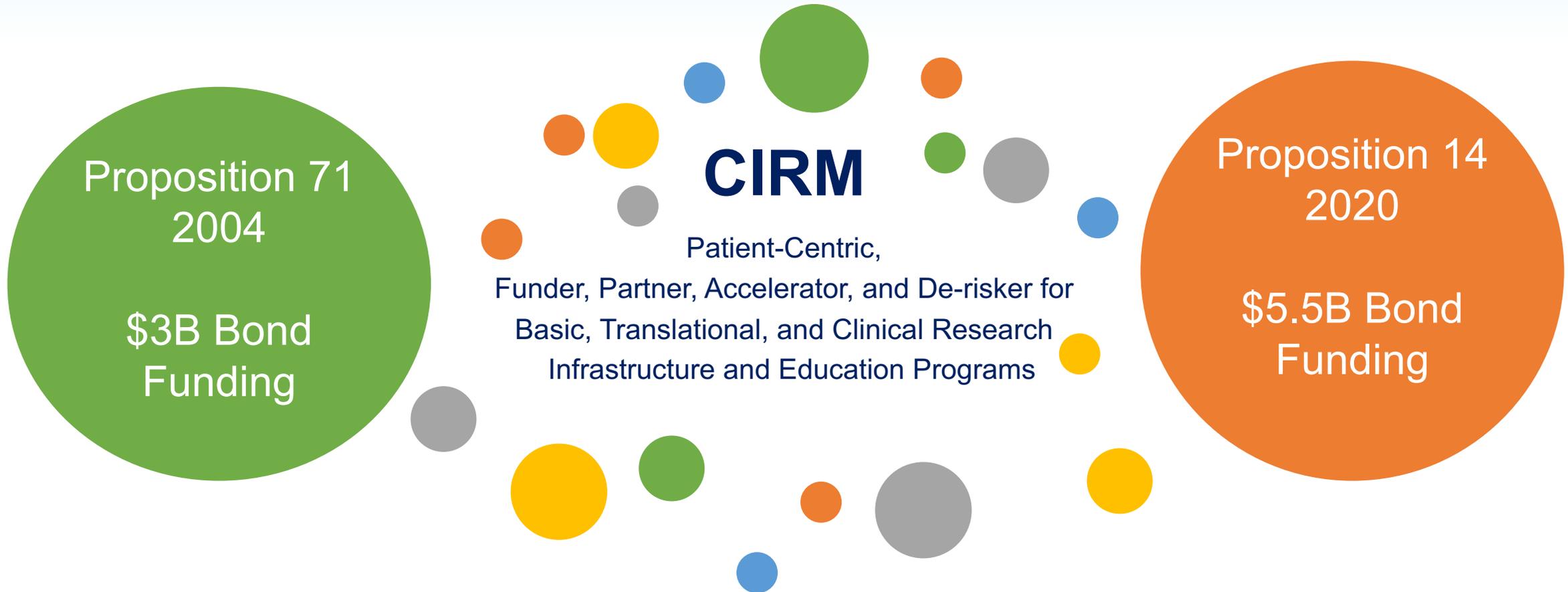


Real Life™

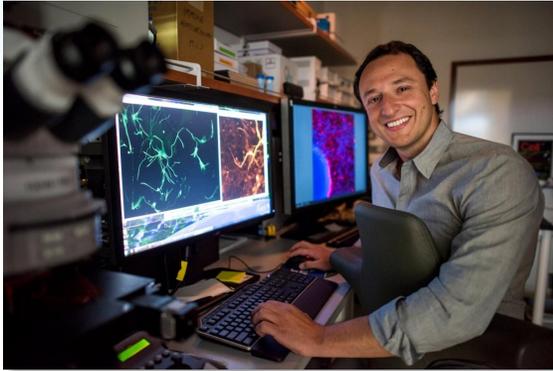
Maria T. Millan, M.D.
President & CEO
December ICOC Meeting
December 14, 2021

CIRM
CALIFORNIA'S STEM CELL AGENCY





- Funded **>1000 projects**
- Advanced stem cell research and therapy development spanning **more than 75 diseases**
- Funded **76 clinical trials with 3200+ patients enrolled**
 - from first-in-human to pivotal phase 3
 - from common diseases to **rare diseases**
 - demonstrated that cures are possible (40 children treated with cell-gene therapy for ADA-SCID)



- Generated **3,000 peer-reviewed publications**
- Funded **12 stem cell research facilities and 17 shared research laboratories**
- Enabled **invention** of research and translational tools
- Built the **largest iPSC research bank with >2,600 cell lines** for modeling
- Created **novel genomic datasets and bioinformatics tools** for stem cell research



- Established **5 Alpha Stem Cell Clinics** that have accelerated execution of 100+ stem cell clinical trials
- Attained **Expedited Pathway Designation** from FDA: **15% of total RMATs**
- Supported **progression of 81 programs into therapy development** via successive CIRM awards
- Accelerated **73% of funded IND-enabling programs** to initiate clinical trials within 2 years



- Trained **over 3,000 students and scholars** to become the future workforce of regenerative medicine
- Stimulated the CA economy with **\$10.7B of gross output and 56,000 new FTE jobs** created during the 2004-2018 period
- Positioned CIRM-funded projects to **attract \$18B+ of industry funding**

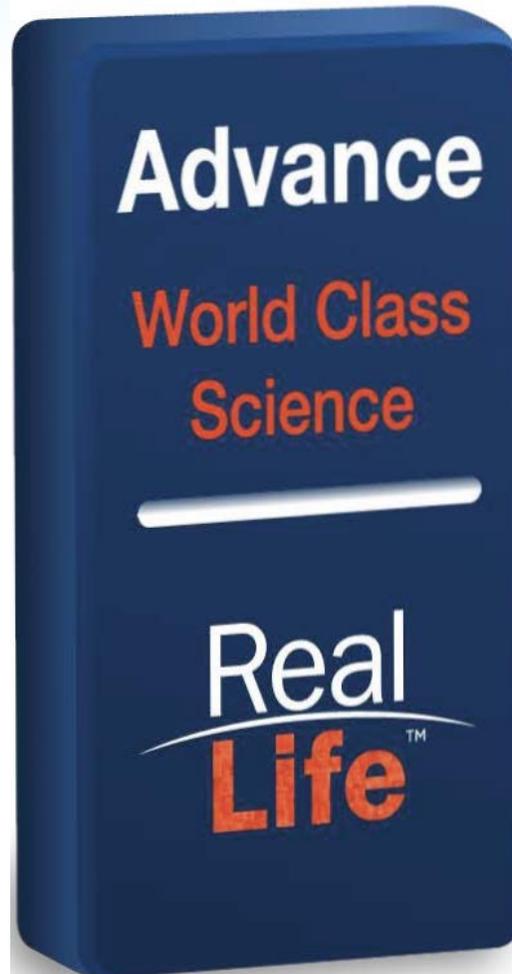


Accelerating world class science to deliver transformative regenerative medicine treatments in an equitable manner to a diverse California and world

The strategy for accomplishing this bold mission will leave a lasting legacy through the development of **novel scientific endeavors, effective healthcare delivery models, and expanded education and training programs.**

The new strategic plan is designed to **enhance**, **organize** and **interconnect** CIRM's proven funding model to achieve the overarching goals.





CIRM will leverage collective scientific knowledge to inspire collaborative research that addresses Californians' unmet medical needs.

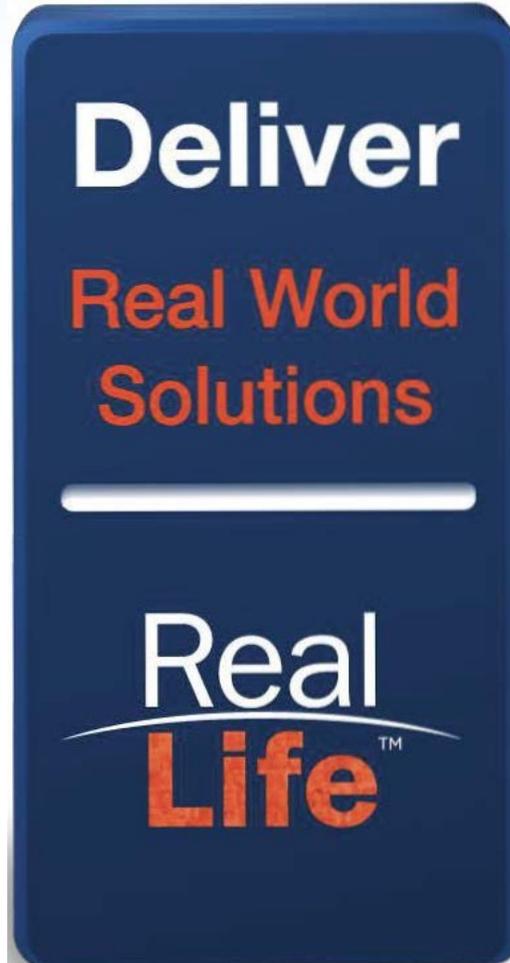
5-year Strategic Goals

- **Continue funding mechanisms** through the existing pillar program, which promote the success of high-risk/high-reward approaches
- **Develop next-generation technology competency hubs**
(One example: Shared labs*)
- **Build knowledge networks** to facilitate data sharing and to foster a culture of open science
(Initially deployed to enable a consortium approach to CNS research**, including neuropsychiatric and neurodevelopmental disorders)

* *Funding for shared labs stipulated in Prop 14*

** *To optimize the \$1.5B investment for CNS-related programs as stipulated in Prop 14*





CIRM will overcome critical bottlenecks to accelerate approval of therapies for all patients.



5-year Strategic Goals

- Optimize CIRM's **clinical trial funding partnership model** to advance more therapies to FDA marketing approval
- Overcome manufacturing hurdles for the delivery of regenerative medicine therapies by building a **public-private manufacturing partnership network**
- Expand **Alpha Clinics** and create **Community Care Centers of Excellence** that support diverse patient participation in the rapidly maturing regenerative medicine landscape

Optimize CIRM's clinical trial funding partnership model to advance more therapies to FDA marketing approval



- Integrated approach of CIRM's Therapeutics Development Program
- Enables partnership with external stakeholders, regulators and payors
- Allows for expansion of CIRM's Advisory Panel program to overcome hurdles to marketing approval and post-market considerations

Overcome manufacturing hurdles for the delivery of regenerative medicine therapies by building a public-private manufacturing partnership network



CIRM-Funded Academic GMP Facility Network



Industry Partners

- Manufacturing Services
- Resources
- Investment and Partnerships



CIRM
CALIFORNIA'S STEM CELL AGENCY

- **Accelerate** and de-risk path to commercialization
- **Advance** standards and quality by design
- **Build** manufacturing leadership and workforce

Expand Alpha Clinics and create Community Care Centers of Excellence that support diverse patient participation in the rapidly maturing regenerative medicine landscape



** Alpha Clinics Expansion and Community Care Centers of Excellence stipulated in Prop 14*

The Alpha Clinics Expansion

- Enable development and support of innovative clinical research models
- Increase access to clinical trials and approved treatments
- Expose basic scientists to clinical research
- Train clinical trial workforce including nurses and clinical research coordinators
- Partner with professional societies to develop regenerative medicine specialty certification/accreditation programs

The Community Care Centers

- Support patient referral, recruitment & access to regenerative medicine clinical trials
- Address the unique needs of the given community
- Leverage the offerings of the Alpha Clinics and CIRM's academic institutions
- Expand engagement and participation of diverse communities in clinical research





CIRM will build inclusive participation opportunities for all stakeholders, from the students to the workforce to the patients.



- Build a **diverse and highly skilled workforce** to support the growing regenerative medicine economy in California
- Deliver a roadmap for **access and affordability** of regenerative medicine for all California patients

- Create multiple on-ramps to develop the next generation of leaders, scientists, clinicians & future workforce
- Incorporate training opportunities within CIRM scientific & infrastructure pillars:
 - Basic research
 - Translational & clinical research
 - Manufacturing sciences
 - Data science
 - Science communication
 - Community engagement



Summary of 5-Year Strategy



- Develop Competency Hubs
- Build Knowledge Networks



- Advance therapies to marketing approval
- Create a manufacturing partnership network
- Expand Alpha Clinics Network
- Create Community Care Centers of Excellence



- Build a diverse and highly skilled workforce
- Deliver a roadmap for access and affordability

Request for Board Action:

Approval of the Proposed CIRM 5-year Strategic Plan (2022-2027)